General

Guideline Title

Eltrombopag for treating chronic immune (idiopathic) thrombocytopenic purpura (review of technology appraisal 205).

Bibliographic Source(s)

National Institute for Health and Care Excellence (NICE). Eltrombopag for treating chronic immune (idiopathic) thrombocytopenic purpura (review of technology appraisal 205). London (UK): National Institute for Health and Care Excellence (NICE); 2013 Jul. 57 p. (Technology appraisal guidance; no. 293).

Guideline Status

This is the current release of the guideline.

This guideline updates a previous version: National Institute for Health and Clinical Excellence (NICE). Eltrombopag for the treatment of chronic immune (idiopathic) thrombocytopenic purpura. London (UK): National Institute for Health and Clinical Excellence (NICE); 2010 Oct. 47 p. (Technology appraisal guidance; no. 205).

Recommendations

Major Recommendations

Note: This guidance replaces National Institute for Health and Care Excellence (NICE) technology appraisal guidance 205 issued in October 2010.

Eltrombopag is recommended as an option for treating adults with chronic immune (idiopathic) thrombocytopenic purpura, within its marketing authorisation (that is, in adults who have had a splenectomy and whose condition is refractory to other treatments, or as a second-line treatment in adults who have not had a splenectomy because surgery is contraindicated), only if:

- Their condition is refractory to standard active treatments and rescue therapies, or
- They have severe disease and a high risk of bleeding that needs frequent courses of rescue therapies and
- The manufacturer provides eltrombopag with the discount agreed in the patient access scheme.

People currently receiving eltrombopag whose disease does not meet the criteria described above should be able to continue treatment until they and their clinician consider it appropriate to stop.

Clinical Algorithm(s)

None provided

Scope

Disease/Condition(s)

Chronic immune (idiopathic) thrombocytopenic purpura

Guideline Category

Assessment of Therapeutic Effectiveness

Treatment

Clinical Specialty

Family Practice

Hematology

Internal Medicine

Intended Users

Advanced Practice Nurses

Nurses

Physician Assistants

Physicians

Guideline Objective(s)

To assess the clinical effectiveness and cost-effectiveness of eltrombopag for the treatment of chronic immune (idiopathic) thrombocytopenic purpura

Target Population

Adult patients with chronic immune (idiopathic) thrombocytopenic purpura

Interventions and Practices Considered

Eltrombopag

Major Outcomes Considered

- Clinical effectiveness:
  - Platelet count (median, response rate, durability of response)
  - Need for rescue treatment or concurrent immune thrombocytopenic purpura (ITP) treatment
  - Symptoms reduction

- Adverse events
- Bleeding events (incidence, severity and outcome)
- Mortality
- Health related quality of life

- Cost-effectiveness

Methodology

Methods Used to Collect/Select the Evidence

Hand-searches of Published Literature (Primary Sources)

Hand-searches of Published Literature (Secondary Sources)

Searches of Electronic Databases

Description of Methods Used to Collect/Select the Evidence

Note from the National Guideline Clearinghouse (NGC): The National Institute for Health and Care Excellence (NICE) commissioned an independent academic centre to perform a systematic literature review on the technology considered in this appraisal and prepare an assessment report. The Evidence Review Group (ERG) report for this technology appraisal was prepared by Aberdeen Health Technology Assessment (HTA) Group (see the "Availability of Companion Documents" field).

Clinical Effectiveness

In the current submission the manufacturer updated the systematic review which they conducted for the previous TA205 submission.

Description of Manufacturer's Search Strategies and Critique

Details of the literature searches undertaken on February 6th and 27th, 2012 are reported in Appendix 2 of the submission report. MEDLINE, MEDLINE In-Process, EMBASE and CENTRAL were searched and were supplemented by searching the conference proceedings of the European Haematology Association and American Society of Hematology for the years 2004 to present. The searches were identical to those undertaken in 2009 for the original submission so were therefore run from January 2009 and any records previously retrieved in the original searches were excluded at the abstract review stage. Other databases such as Science Citation Index, Cumulative Index to Nursing and Allied Health (CINAHL) and Biosis would have been appropriate to search but the included sources were the main ones and as such should have provided adequate coverage of the literature.

The full search strategies that were used are provided and were therefore reproducible. The approach adopted was to carry out one search to find all relevant clinical and quality of life information on the intervention and comparators included in the systematic review. The searches were constructed using three sets of terms: (a) immune thrombocytopenic purpura (ITP) terms, (b) intervention/comparator terms, and (c) methodology terms. These were correctly combined using the Boolean operator OR for each set of terms. Then the summaries of each set were combined using AND. Both controlled vocabulary terms and free text terms were used but some key terms were omitted which may have compromised the sensitivity of the search. For example, free text searching did not always include common variations. Most notable omissions were variation for "thrombocytopenic" (thrombocytopaenic and thrombocytopenia) and "romiplostim" (nplate, AMG 532, AMG 531 and remiplistim).

The methodology parts of the MEDLINE and EMBASE search strategies were the weakest sections and were difficult to follow. This was largely due to the duplicate use of some controlled vocabulary terms both as single terms and as part of higher order exploded terms. For example in MEDLINE, "Controlled Clinical Trial/" is captured by "exp Clinical Trial/" and "Prospective Studies/" by "exp Cohort Studies/". Some appropriate terms were excluded" for example the MeSH term "Comparative Study/", and EMTREE terms "Controlled Study/" and "Retrospective Study/". The strategy would also have benefited from additional methodology-related text terms. In particular, the term "retrospective" was omitted even though retrospective studies were listed in the inclusion criteria.

The search strategy used in CENTRAL also included a methodology section. This seemed unnecessary since this database consists mostly of trials and inclusion risked compromising the sensitivity of the search.

Due to concerns over the sensitivity of the manufacturer's clinical effectiveness search searches, the ERG undertook independent searches for eltrombopag and the comparators. MEDLINE, MEDLINE In-Process and EMBASE databases were searched. The eltrombopag search comprised ITP-related and eltrombopag terms only to maximise the sensitivity of the search. The multifile search in MEDLINE and EMBASE for comparators was similar to the structure of the manufacturer's search but included additional controlled vocabulary and text terms. The terms used relating to methodology included those used in the Cochrane Highly Sensitive randomised controlled trial (RCT) filter and a published filter selective for comparative and case series studies. Additional details are provided in Appendix 1 of the ERG report.

Inclusion Criteria

The inclusion criteria used in the systematic review of clinical effectiveness are tabulated in Table 1 of the ERG report. In addition, refer to Section 4 of the ERG report for the ERG's comments regarding the manufacturer's inclusion/exclusion criteria.

Cost-Effectiveness

Description of Manufacturer's Search Strategies and Critique

The searches for cost-effectiveness data were undertaken in MEDLINE, MEDLINE In-process, EMBASE, Econlit, National Health Service Economic Evaluation Database (NHS EED) and Health Economic Evaluation Database (HEED) on February 6th, 2012 and are provided in full in the submission. The MEDLINE and EMBASE strategies combined the same ITP terms as was used for the clinical effectiveness combined with (AND) a variety of very broad cost and economic terms. As for CENTRAL, the NHS EED strategy also included methods search terms which again seems unnecessary since this is a database of economic evaluations so inclusion of such terms may reduce sensitivity.

Separate searches for utilities and quality of life information were undertaken in MEDLINE and EMBASE on March 16, 2012 using additional quality of life (QoL) terms to those included in the clinical effectiveness search. These searches are reproduced in full in the submission. The methods section of the searches used a comprehensive selection of both controlled vocabulary and text terms. All searches were limited to 2009 - onwards to update the systematic review conducted for the previous eltrombopag submission.

The ERG is satisfied with the cost-effectiveness search strategies developed by the manufacturer.

Inclusion and Exclusion Criteria

Studies were included if they enrolled adults with ITP as a primary diagnosis with a median/mean platelet counts <30 x 10^9/L at baseline. Studies where patients had a higher platelet count at baseline, were included only if a proportion of patients had a platelet count <30 x 10^9/L. Non-English language studies as well as studies published as conference abstracts were excluded.

Results and Conclusions

The manufacturer did not identify any suitable study to be included in the cost-effectiveness systematic review.

Number of Source Documents

Clinical Effectiveness

- 11 eltrombopag studies of which four were randomised controlled trials (RCTs)
- Four romiplostim studies of which two were RCTs
- 37 non thrombopoietin receptor agonist (non-TPO-RA) studies of which six were RCTs

Cost-Effectiveness

- No relevant economic evaluations were identified
- The manufacturer submitted an economic model

Methods Used to Assess the Quality and Strength of the Evidence

Expert Consensus

Rating Scheme for the Strength of the Evidence

Not applicable

Methods Used to Analyze the Evidence

Meta-Analysis

Review of Published Meta-Analyses

Systematic Review with Evidence Tables

Description of the Methods Used to Analyze the Evidence

Note from the National Guideline Clearinghouse (NGC): The National Institute for Health and Care Excellence (NICE) commissioned an independent academic centre to perform a systematic literature review on the technology considered in this appraisal and prepare an assessment report. The Evidence Review Group (ERG) report for this technology appraisal was prepared by Aberdeen Health Technology Assessment (HTA) Group (see the "Availability of Companion Documents" field).

Clinical Effectiveness

Critique of Data Extraction

Details on data extraction were provided in the supplementary information sent by the manufacturer in response to the ERG clarification letter.

Few details were provided on how data were extracted for the original review. The report mentions the use of a pre-determined data extraction table. However, no information was provided on who actually extracted data from included studies. Ideally data extraction would have been performed independently by two reviewers, and any disagreements would have been resolved by discussion.

More details were provided on how data extraction was performed for the updated review. The ERG considers that all of the listed study and patient characteristics and outcomes are relevant. However, it was also unclear from this report who carried out data extraction.

Quality Assessment

Details on the assessment of the methodological quality of included studies were provided in both the manufacturer's submission and the supplementary information sent by the manufacturer in response to the ERG clarification letter.

The manufacturer appraised the methodological quality of randomised controlled trials (RCTs) (except those non-thrombopoietin receptor agonist [non-TPO-RA] trials which were excluded *post hoc*). Quality assessment was not performed on studies reported as conference proceedings or on non-randomised studies. Whilst the ERG considers it appropriate not to attempt quality assessment on conference abstracts, to avoid appraising non randomised evidence is less justifiable.

Details on who performed quality assessment were provided in the two supplementary systematic review reports. Two reviewers independently assessed the risk of bias of each included study and final agreement was reached by consensus. The ERG considers this strategy appropriate. The tool used for quality assessment was akin to the Cochrane Collaboration risk of bias tool and was judged appropriate by the ERG.

Eltrombopag RCTs

Overall, the quality of the eltrombopag RCTs was good. Randomisation was achieved using an in-house randomisation system using a computerised schedule. Investigators and assessors were blind to treatment status and blinding was maintained in patients using matching placebo tablets. The manufacturer stated that participants could be unblinded when knowledge of treatment status was necessary for the care of the subject. However, it is not clear if any patients were unblinded, and if so if these patients continued in the trial. It is also unclear how blinding was maintained in patients who had large platelet responses to eltrombopag, particularly if eltrombopag treatment was interrupted or discontinued because of a high platelet level.

Romiplostim RCTs

The quality of the two romiplostim RCTs was good. Patients were randomly assigned to either romiplostim or placebo using an interactive voice response system and a random allocation sequence generated by Amgen. Patient and physicians were blind to treatment status. Blinding was maintained using identical vials for romiplostim and placebo. The manufacturer rated all of the items on the quality assessment tool as "low risk". The ERG agrees with this assessment.

Non-TPO-RA RCTs

Information related to the quality assessment of the non-TPO-RA RCTs was provided in the supplementary material (updated systematic review details) sent by the manufacturer in response to the ERG clarification letter. This material was not included, or referred to, in the main submission document. Only the methodological quality of the included RCTs was critically appraised (six trials) but not the quality of the non-randomised studies (31 non-TPO-RA studies).

Overall, the manufacturer considered the quality of the six RCTs to be of "low risk". The ERG did not validate the quality assessment of these studies.

Evidence Synthesis

Quantitative synthesis of eltrombopag evidence as regards to its clinical effectiveness consisted of: i) a direct comparison between eltrombopag and placebo, ii) an indirect comparison between eltrombopag and romiplostim, and iii) a systematic review of non-TPO-RA interventions.

The comparison between eltrombopag and placebo used data from the three included eltrombopag RCTs. A fourth eltrombopag RCT was initially identified as potentially relevant but subsequently excluded because based on a Japanese patient population. Reasons for excluding the Tomiyama trial were not entirely justifiable and the manufacturer could have attempted to include it in further analyses, where appropriate. Data on platelet count $\geq 50 \times 10^9$/L at day 43 from TRA100773A, TRA100773B, and RAISE trials were combined in a meta-analysis. Although data for bleeding rates and quality of life were also available, these data were not meta-analysed.

An indirect comparison between eltrombopag and romiplostim was carried out using data from the RAISE trial and the two romiplostim RCTs, with placebo as a common comparator. Data from TRA100773A and TRA100773B were not included in the indirect comparison. In a response to an ERG query, the manufacturer explained that TRA100773A and TRA100773B could not be included in the analysis of durable platelet response, as this would require at least eight weeks of treatment data. They further argued that since a small number of clinically significant bleeds were observed in TRA100773A and TRA100773B, assessment of bleeding events in the indirect comparison would have been of little clinical value. The ERG agrees with this interpretation.

The efficacy of non-TPO-RA interventions versus eltrombopag was assessed by means of a systematic review of non-TPO-RA studies (mainly observational studies), which met both the original inclusion criteria and the more stringent *post hoc* criteria. Three main outcomes were assessed: platelet response, time to response and duration of response. Simple weighted averages were used for all outcomes to derive pooled results for each included non-TPO-RA comparator, regardless of the outcome definitions used. Analysis of bleeding rates was not conducted. Since the included non-TPO-RA studies were non-randomised and highly heterogeneous and no direct comparisons with eltrombopag were possible, the observed results are likely to be prone to bias and should therefore be interpreted with extreme caution.

The ERG assessed the methodological quality of the manufacturer's updated systematic review of clinical effectiveness using the Centre for Reviews and Dissemination (CRD) criteria (see Table 7 of the ERG report). The methodological quality of the review was mixed. In particular, weaknesses were noticed in the development of the literature searches and in the quality assessment of included studies.

Refer to Section 4 of the ERG report for additional information.

Cost-Effectiveness

Comparison of Economic Submission with NICE Reference Case

See Table 26 of the ERG report for a comparison of the manufacturer's economic submission with the NICE reference case checklist.

Model Structure

The model structure adopts a 4 week cycle length (see Figure 5 of the ERG report). A cohort of patients starting a treatment may respond in the 1st, 2nd, 3rd or 4th cycle, the cycle of response being treatment specific. Those in response have a treatment specific probability of loss of response each cycle. The model also contains the facility for a proportion of those in response to receive rescue therapy, though for these patients rescue therapy only incurs costs.

Those not responding become long term non-responders off treatment. These patients may also receive rescue therapy, which may result in a temporary response of one cycle duration. During each cycle a proportion of long term non-responders exit this state and move on to other treatments further down the treatment sequence.

Rate of rescue treatment, rates of non-severe bleeds treated as outpatients and rates of severe bleeds treated as inpatients are differentiated by

response status, with responders experiencing lower rates than non-responders. These lead on to differential mortality risks.

Refer to Section 5 of the ERG report for additional information on cost-effectiveness analysis.

Methods Used to Formulate the Recommendations

Expert Consensus

Description of Methods Used to Formulate the Recommendations

Considerations

Technology appraisal recommendations are based on a review of clinical and economic evidence.

Technology Appraisal Process

The National Institute for Health and Care Excellence (NICE) invites "consultee" and "commentator" organisations to take part in the appraisal process. Consultee organisations include national groups representing patients and carers, the bodies representing health professionals, and the manufacturers of the technology under review. Consultees are invited to submit evidence during the appraisal and to comment on the appraisal documents.

Commentator organisations include manufacturers of the products with which the technology is being compared, the National Health Service (NHS) Quality Improvement Scotland and research groups working in the area. They can comment on the evidence and other documents but are not asked to submit evidence themselves.

NICE then commissions an independent academic centre to review published evidence on the technology and prepare an "assessment report". Consultees and commentators are invited to comment on the report. The assessment report and the comments on it are then drawn together in a document called the evaluation report.

An independent Appraisal Committee then considers the evaluation report. It holds a meeting where it hears direct, spoken evidence from nominated clinical experts, patients and carers. The Committee uses all the evidence to make its first recommendations, in a document called the "appraisal consultation document" (ACD). NICE sends all the consultees and commentators a copy of this document and posts it on the NICE Web site. Further comments are invited from everyone taking part.

When the Committee meets again it considers any comments submitted on the ACD; then it prepares its final recommendations in a document called the "final appraisal determination" (FAD). This is submitted to NICE for approval.

Consultees have a chance to appeal against the final recommendations in the FAD. If there are no appeals, the final recommendations become the basis of the guidance that NICE issues.

Who Is on the Appraisal Committee?

NICE technology appraisal recommendations are prepared by an independent committee. This includes health professionals working in the NHS and people who are familiar with the issues affecting patients and carers. Although the Appraisal Committee seeks the views of organisations representing health professionals, patients, carers, manufacturers and government, its advice is independent of any vested interests.

Rating Scheme for the Strength of the Recommendations

Not applicable

Cost Analysis

Summary of Appraisal Committee's Key Conclusions on Cost-Effectiveness

Availability and Nature of Evidence

The manufacturer presented three economic analyses: a base case, an alternative evaluation and a scenario analysis. In all three analyses, the

manufacturer compared eltrombopag with a pathway of standard care alone, and separately with a pathway of romiplostim plus standard care.

The Committee agreed that the alternative evaluation represented the most valid analysis because the modelling applied data derived directly from the pivotal trials of eltrombopag and the manufacturer's own systematic review.

Uncertainties Around and Plausibility of Assumptions and Inputs in the Economic Model

The Committee considered that there was no sufficiently robust cost-effectiveness evidence to make a recommendation for eltrombopag compared with the pathway of standard care alone because the model incorporated data based on the manufacturer's indirect comparison of treatments used in standard care that, in the Committee's opinion, lacked methodological rigor.

The Committee agreed that romiplostim is likely to be more clinically effective than eltrombopag, that it was appropriate to use the Short Form 6 dimensions (SF-6D) utility data collected from RAISE and EXTEND trials, a lower romiplostim dose and a lower administration cost for romiplostim, and to exclude anti-D. The Committee acknowledged that the incremental cost-effectiveness ratios (ICERs), even those reflecting its favoured parameters and assumptions, are associated with considerable uncertainty.

The Committee noted that there was no information available on bleeding and rescue rates observed in clinical practice. It accepted that, if these rates are higher than those applied in the model, this would have an impact on the ICERs in favour of romiplostim, but it would be extremely unlikely to affect the relative cost-effectiveness of eltrombopag and romiplostim to a degree where the Committee would change its recommendations.

Incorporation of Health-Related Quality-of-Life Benefits and Utility Values/Have any Potential Significant and Substantial Health-Related Benefits Been Identified That Were not Included in the Economic Model, and how Have They Been Considered?

The Committee noted that the manufacturer did not use the health-related quality-of-life data collected from RAISE and EXTEND. The Committee concluded that, of the utility data available, the SF-6D data provided by the manufacturer were the most appropriate to use within the alternative evaluation.

The Committee considered that the EQ-5D data obtained from using a particular mapping algorithm would be associated with further uncertainty and, in the absence of other EQ-5D data, the Committee concluded that the SF-6D data provided by the manufacturer were the most appropriate to use within the alternative evaluation.

The Committee noted that adequate treatment could psychologically benefit people with chronic idiopathic thrombocytopenic purpura (ITP) and their families by reducing anxiety and enabling them to lead more normal lives. The Committee agreed that these benefits may not be fully captured in the calculation of the quality-adjusted life year (QALY).

Are There Specific Groups of People for Whom the Technology Is Particularly Cost-Effective?

The Committee considered the cost effectiveness of eltrombopag compared with the pathway of standard care plus romiplostim in the alternative evaluation. It noted that the results of this comparison would apply only to people with severe chronic ITP and a persistent high risk of bleeding (that is, people for whom romiplostim is recommended in NICE technology appraisal guidance 221 for romiplostim).

What Are the Key Drivers of Cost-Effectiveness?

The key driver of cost-effectiveness is the relative effect size of eltrombopag and romiplostim. The Committee did not agree with the manufacturer's assumption that eltrombopag and romiplostim were equally effective, and so considered the sensitivity analyses in which romiplostim was more effective than eltrombopag.

Most Likely Cost-Effectiveness Estimate (Given as an ICER)

The Committee considered the analysis that mirrored its preferred assumptions and parameters. It noted that the resulting ICERs for eltrombopag compared with romiplostim were £389,000 saved per QALY lost for patients who had had a splenectomy and £271,000 saved per QALY lost for patients who had not had a splenectomy.

Refer to Sections 3 and 4 in the original guideline document for details of the economic analyses provided by the manufacturer, the Evidence Review Group (ERG) comments, and the Appraisal Committee considerations.

Method of Guideline Validation

External Peer Review

Description of Method of Guideline Validation

Consultee organisations from the following groups were invited to comment on the draft scope, Assessment Report and the appraisal consultation document (ACD) and were provided with the opportunity to appeal against the final appraisal determination.

- Manufacturer/sponsors
- Professional/specialist and patient/carer groups
- Commentator organisations (without the right of appeal)

In addition, individuals selected from clinical expert and patient advocate nominations from the professional/specialist and patient/carer groups were also invited to comment on the ACD.

Evidence Supporting the Recommendations

Type of Evidence Supporting the Recommendations

The type of evidence supporting the recommendations is not specifically stated.

The Appraisal Committee considered evidence submitted by the manufacturer of eltrombopag and a review of this submission by the Evidence Review Group.

Benefits/Harms of Implementing the Guideline Recommendations

Potential Benefits

Appropriate use of eltrombopag for the treatment of chronic immune (idiopathic) thrombocytopenic purpura

Potential Harms

The summary of product characteristics lists the following adverse reactions for eltrombopag as being common (1 or more patient in every 100 and fewer than 1 patient in every 10) or very common (1 or more patient in every 10): psychiatric disorders (insomnia), nervous system disorders (headache and paraesthesia), eye disorders (cataract and dry eye), gastrointestinal disorders (nausea, diarrhoea, constipation and upper abdominal pain), hepatobiliary disorders (increased alanine aminotransferase, increased aspartate aminotransferase, increased blood bilirubin and hyperbilirubinaemia, and abnormal hepatic function), skin and subcutaneous tissue disorders (rash, pruritus and alopecia), musculoskeletal and connective tissue disorders (arthralgia, myalgia, muscle spasm and bone pain), and general disorders (fatigue and peripheral oedema).

For full details of adverse effects and contraindications, see the summary of product characteristics available at http://emc.medicines.org.uk/ .

Qualifying Statements

Qualifying Statements

- This guidance represents the views of National Institute for Health and Care Excellence (NICE) and was arrived at after careful consideration of the evidence available. Healthcare professionals are expected to take it fully into account when exercising their clinical judgement. However, the guidance does not override the individual responsibility of healthcare professionals to make decisions appropriate to the circumstances of the individual patient, in consultation with the patient and/or guardian or carer.
- Implementation of this guidance is the responsibility of local commissioners and/or providers. Commissioners and providers are reminded

that it is their responsibility to implement the guidance, in their local context, in light of their duties to have due regard to the need to eliminate unlawful discrimination, advance equality of opportunity and foster good relations. Nothing in this guidance should be interpreted in a way that would be inconsistent with compliance with those duties.

Implementation of the Guideline

Description of Implementation Strategy

- Section 7(6) of the National Institute for Health and Care Excellence (NICE) (Constitution and Functions) and the Health and Social Care Information Centre (Functions) Regulations 2013 requires clinical commissioning groups, National Health Service (NHS) England and, with respect to their public health functions, local authorities to comply with the recommendations in this appraisal within 3 months of its date of publication.
- When NICE recommends a treatment "as an option", the NHS must make sure it is available within the period set out in the paragraph above. This means that, if a patient has chronic immune (idiopathic) thrombocytopenic purpura and the doctor responsible for their care thinks that eltrombopag is the right treatment, it should be available for use, in line with NICE's recommendations.
- The Department of Health and the manufacturer have agreed that eltrombopag will be available to the NHS with a patient access scheme which makes eltrombopag available with a discount. The size of the discount is commercial in confidence. It is the responsibility of the manufacturer to communicate details of the discount to the relevant NHS organisations. Any enquiries from NHS organisations about the patient access scheme should be directed to the manufacturer's customer contact centre on 0800 221 441.
- NICE has developed tools to help organisations put this guidance into practice (listed below). These are available on the NICE Web site (http://guidance.nice.org.uk/TA293).
    - A costing statement explaining the resource impact of this guidance.
    - Audit support for monitoring local practice.

Implementation Tools

Audit Criteria/Indicators

Foreign Language Translations

Patient Resources

Resources

For information about availability, see the *Availability of Companion Documents* and *Patient Resources* fields below.

Institute of Medicine (IOM) National Healthcare Quality Report Categories

IOM Care Need

Getting Better

Living with Illness

IOM Domain

Effectiveness

Patient-centeredness

Identifying Information and Availability

Bibliographic Source(s)

National Institute for Health and Care Excellence (NICE). Eltrombopag for treating chronic immune (idiopathic) thrombocytopenic purpura (review of technology appraisal 205). London (UK): National Institute for Health and Care Excellence (NICE); 2013 Jul. 57 p. (Technology appraisal guidance; no. 293).

Adaptation

Not applicable: The guideline was not adapted from another source.

Date Released

2010 Oct (revised 2013 Jul)

Guideline Developer(s)

National Institute for Health and Care Excellence (NICE) - National Government Agency [Non-U.S.]

Source(s) of Funding

National Institute for Health and Care Excellence (NICE)

Guideline Committee

Appraisal Committee

Composition of Group That Authored the Guideline

Committee Members: Dr Amanda Adler (*Chair*), Consultant Physician, Addenbrooke's Hospital; Professor Ken Stein (*Vice Chair*), Professor of Public Health, Peninsula Technology Assessment Group (PenTAG), University of Exeter; Dr Ray Armstrong, Consultant Rheumatologist, Southampton General Hospital; Dr Jeff Aronson, Reader in Clinical Pharmacology, University Department of Primary Health Care, University of Oxford; Dr Peter Barry, Consultant in Paediatric Intensive Care, Leicester Royal Infirmary; Professor John Cairns, Professor of Health Economics Public Health and Policy, London School of Hygiene and Tropical Medicine; David Chandler, Lay Member; Mark Chapman, Health Economics and Market Access Manager, Medtronic UK; Professor Fergus Gleeson, Consultant Radiologist, Churchill Hospital, Oxford; Professor Daniel Hochhauser, Consultant in Medical Oncology; Dr Neil Iosson, General Practitioner; Anne Joshua, Associate Director of Pharmacy, NHS Direct; Terence Lewis, Lay member; Professor Ruairidh Milne, Director of Strategy and Development and Director for Public Health Research at the National Institute for Health Research (NIHR) Evaluation, Trials and Studies Coordinating Centre at the University of Southampton; Dr Rubin Minhas, General Practitioner and Clinical Director, BMJ Evidence Centre; Dr Elizabeth Murray, Reader in Primary Care, University College London; Dr Peter Norrie, Principal Lecturer in Nursing, DeMontfort University; Dr Sanjeev Patel, Consultant Physician & Senior Lecturer in Rheumatology, St Helier University Hospital; Dr John Pounsford, Consultant Physician, Frenchay Hospital, Bristol; Dr Danielle Preedy, Lay member; Alun Roebuck, Consultant Nurse in Critical and Acute Care, United Lincolnshire NHS Trust; Roderick Smith, Finance Director, West Kent Primary Care Trust; Cliff Snelling, Lay Member; Marta Soares, Research Fellow, Centre for Health Economics, University of York; Professor Andrew Stevens, Professor of Public Health, Department of Public Health and Epidemiology, University of Birmingham; Dr Nerys Woolacott, Senior Research Fellow, Centre for Health Economics, University of York

Financial Disclosures/Conflicts of Interest

Committee members are asked to declare any interests in the technology to be appraised. If it is considered there is a conflict of interest, the member is excluded from participating further in that appraisal.

Guideline Status

This is the current release of the guideline.

This guideline updates a previous version: National Institute for Health and Clinical Excellence (NICE). Eltrombopag for the treatment of chronic immune (idiopathic) thrombocytopenic purpura. London (UK): National Institute for Health and Clinical Excellence (NICE); 2010 Oct. 47 p. (Technology appraisal guidance; no. 205).

Guideline Availability

Electronic copies: Available from the National Institute for Health and Care Excellence (NICE) Web site .

Availability of Companion Documents

The following are available:

- Cummins E, Fielding S, Scott N, Rothnie K, Crowther M, Fraser C, Brazzelli M. Eltrombopag for the treatment of chronic immune thrombocytopenic purpura: a single technology appraisal. Aberdeen (Scotland): Aberdeen Health Technology Assessment (HTA) Group; 2012 Oct 19. 184 p. Electronic copies: Available in Portable Document Format (PDF) from the National Institute for Health and Care Excellence (NICE) Web site .
- The treatment of chronic immune (idiopathic) thrombocytopenic purpura. Clinical audit tool. London (UK): National Institute for Health and Care Excellence (NICE); 2013 Jul. (Technology appraisal guidance; no. 293). Electronic copies: Available from the NICE Web site .
- Eltrombopag for treating chronic immune (idiopathic) thrombocytopenic purpura (review of technology appraisal 205). Costing statement. London (UK): National Institute for Health and Care Excellence (NICE); 2013 Jul. 6 p. (Technology appraisal guidance; no.293). Electronic copies: Available in PDF from the NICE Web site .

Patient Resources

The following is available:

- Eltrombopag for chronic immune (idiopathic) thrombocytopenic purpura. Information for the public. London (UK): National Institute for Health and Care Excellence (NICE); 2013 Jul. 6 p. (Technology appraisal guidance; no. 293). Electronic copies: Available in Portable Document Format (PDF) from the National Institute for Health and Care Excellence (NICE) Web site . Also available in Welsh from the NICE Web site .

Please note: This patient information is intended to provide health professionals with information to share with their patients to help them better understand their health and their diagnosed disorders. By providing access to this patient information, it is not the intention of NGC to provide specific medical advice for particular patients. Rather we urge patients and their representatives to review this material and then to consult with a licensed health professional for evaluation of treatment options suitable for them as well as for diagnosis and answers to their personal medical questions. This patient information has been derived and prepared from a guideline for health care professionals included on NGC by the authors or publishers of that original guideline. The patient information is not reviewed by NGC to establish whether or not it accurately reflects the original guideline's content.

NGC Status

This NGC summary was completed by ECRI Institute on May 20, 2011. This summary was updated by ECRI Institute on October 31, 2013.

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